Disease Team Therapy Development Research Award Webinar

CIRM conducted a webinar on September 9, 2011 to provide information regarding the RFA 10-05 Supplement and the Research Award application process and address questions from applicants. Archived materials from this webinar are available below for your reference.

Webinar Resources

Slides

- Download the slide presentation from the webinar.

Video

- Listen to the webinar with slides.

FAQs

Regulatory questions

1. For IND-enabling studies, is it required to have a pre-pre-IND meeting with the FDA before the submission of the Part II grant proposal?
2. How do we handle it if there is a change in trial design based on FDA recommendations?

CIRM portfolio questions

3. What does CIRM consider "a substantially comparable" approach/intervention? (This question refers to a statement in the RFA that the project proposed in the research award be substantially comparable to that proposed in the Planning Award)
4. What are "unique and compelling" reasons for submitting a proposal that is similar to one already represented in CIRM's translational portfolio?
5. Will CIRM allow for-profits to receive awards for a disease target if other entities have already received awards for the same disease target but a different product? And if so, what criteria must be met?

PI and team composition questions

6. Can companies join a team as a partner? What's the requirement?
7. Can the Principal Investigator be located outside of California but providing at least 30% effort to the project?

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8. If the grant reviewer specifically recommended expertise that exists only outside of California, can the consultant budget be expanded to include that additional consultant?

Exemption from the planning award requirement

9. Can you clarify whether only for-profit entities can ask for exception under section V.D?
10. Is there a limit to the number of exceptions that a for-profit can submit?
11. Is it possible that decisions for an exception will come before Nov 15?

Other sources of funding

12. For securing additional funds by for-profit entities, do the secured funds have to be spent in parallel with the grant award or can the secured funds be spent after the CIRM funds?

13. If a for-profit is selected for an award, they will be encouraged to raise 50% of the project costs and the other 50% is a loan to the company?

Clinical trial questions

14. Is an Investigator Brochure (Part G) required of all applicants requesting to conduct clinical trials?
15. We have a rather mature study that is in the process of filing an IND but we would like to obtain additional funding to enroll more patients. Do you think we can apply for this grant?
16. Would we be eligible to apply if we have completed enrollment for Phase 1, but the trials have to be finished?
17. What if we plan to file an IND and then begin a trial in Year 3 of the project?

Grant review and awarding questions

18. Will the Grants Working Group for the Disease Team Research Awards be the same as that which reviewed the Planning Award applications?
19. Can for-profit organizations qualify for grants instead of loans?
20. Our timeline for IND submission has been accelerated since submitting the Planning Award. What are the implications for obtaining funding for IND-enabling activities that have already been completed?
21. Can a for-profit hold more than one Disease Team award?
22. If I have one Planning Award, can I submit two proposals if we use different PIs?
23. Will CIRM limit the number of awards to a for-profit company in this cycle?

Regulatory questions

1. For IND-enabling studies, is it required to have a pre-pre-IND meeting with the FDA before the submission of the Part II grant proposal?

Although it is not a requirement, evidence of having completed a pre-pre-IND meeting is considered within the review criteria. Completion of a pre-pre-IND meeting is one indicator of readiness to begin IND-enabling studies that CIRM's Grants Working Group will consider. If you have held your pre-pre-IND please share that information with your application if possible. Typically only one pre-pre-IND meeting is granted so if you are not ready for discussions with the FDA, don't hold a pre-pre-IND meeting for the purposes of this application if it would not be productive.

2. How do we handle it if there is a change in trial design based on FDA recommendations?

The objective of this RFA is to advance preclinical and/or early clinical development. If your project is funded, your team will be in regular contact with your assigned Science Officer and providing quarterly project updates. If a change in trial design for a funded project is recommended by the FDA, CIRM will work with you to enable the project to continue to move forward. If recommended changes will significantly impact the timeline and/or budget of the project, those will have to be addressed on a case-by-case basis. That is based on the assumption that the FDA-recommendation occurs after a project is successfully funded. If changes in trial design were recommended by the FDA in between submission of your Planning Award and Research Award applications, CIRM will also consider allowing the modified project proposal to move forward with appropriate explanation and rationale for the differences between the Planning Award proposal and the Research Award application.

CIRM Portfolio questions

3. What does CIRM consider "a substantially comparable" approach/intervention? (This question refers to a statement in the RFA that the project proposed in the research award be substantially comparable to that proposed in the Planning Award)

The therapeutic candidate/approach should be similar to the approach/intervention that was proposed in the Planning Award and reviewed by the Grants Working Group. If there is a reason why you need to take a different approach in your Research Award application than that which was described in the Planning Award, you should clearly explain the rationale. CIRM would want to discuss specific questions regarding taking a different approach than what was reviewed and approved in the Planning Award with applicants in advance of submission of the Research Award application.

4. What are "unique and compelling" reasons for submitting a proposal that is similar to one already represented in CIRM's translational portfolio?

To generalize this question, if you see a funded project for the same clinical indication using a similar approach in CIRM's Translational Portfolio, that should not discourage you from applying. But you should provide a strong rationale for your approach and particularly address why your approach may be better than alternatives. All proposals will be reviewed by the GWG based on the criteria in the RFA in Section VII.

5. Will CIRM allow for-profits to receive awards for a disease target if other entities have already received awards for the same disease target but a different product? And if so, what criteria must be met?

In establishing the scientific merit of applications, the same criteria (Section VII of the RFA) apply to all applicants and proposals, whether from a for-profit or not-for-profit institution. Portfolio decisions may be a part of the programmatic discussion when either the GWG or the ICOC considers applications. We direct applicants to the CIRM Translational Portfolio so that you are aware of what CIRM is currently funding.

PI and Team Composition Questions

6. Can companies join a team as a partner? What's the requirement?

Yes, companies can participate in a disease team and CIRM encourages public/private partnerships, especially if the industrial partner has a specific technology or expertise that will help advance the project. It would require your company identifying a particular investigator or team with a project proposal of mutual interest and work with that Principal Investigator in developing the proposal.

7. Can the Principal Investigator be located outside of California but providing at least 30% effort to the project?

Eligibility is discussed in Section V of the RFA. California residency is not a requirement for a PI. Since the work must be performed in California the PI must be in-state for the 30% effort contributed to the award.

Eligible expenses

8. If the grant reviewer specifically recommended expertise that exists only outside of California, can the consultant budget be expanded to include that additional consultant?

CIRM Grant's Administration Policy (GAP), which is available on our website, limits expense on subcontracts or consulting agreements with individuals or organizations outside the state of California to $15,000 per subcontract per budget period, and $25,000 per budget period in aggregate. For amounts above those limits, applicants must seek and obtain Prior Approval during the administrative review that occurs before an approved grant is funded. There is no guarantee that approval will be granted, particularly if the work could be completed in California. In the example posed in this question, the need for additional expertise is identified in the pre-award process so you would need to provide justification to exceed the allowable maximum. Circumstances such as the one posed in this question should be discussed with CIRM on a case-by-case basis.

Exemption from the Planning Award Requirement

9. Can you clarify whether only for-profit entities can ask for exception under section V.D?

The intent of the exceptions pathway was targeted to for-profits, or to recipients of the Disease Team 1 award whom had already filed their IND for the same therapeutic candidate intended for the clinical trial, since they would be the most relevant audience who would not require a planning award. But CIRM will consider on a case-by-case basis exception requests submitted by non-profit entities.

10. Is there a limit to the number of exceptions that a for-profit can submit?

No, but each submission from the same for-profit entity must have a unique Principal Investigator.

11. Is it possible that decisions for an exception will come before Nov 15?

Exception requests are due by October 4th, 2011. It is possible that decisions to these requests may occur earlier, but no later than November 15th. CIRM will work to respond as quickly as possible. We need to ensure adequate time to review those requests so it will depend upon the number received.

Other sources of funding

12. For securing additional funds by for-profit entities, do the secured funds have to be spent in parallel with the grant award or can the secured funds be spent after the CIRM funds?

Funds would be spent in parallel as appropriate, depending upon what activities the respective funds are budgeted for. The intention is that the matching contribution from other resources will leverage CIRM funds for maximal benefit.

13. If a for-profit is selected for an award, they will be encouraged to raise 50% of the project costs and the other 50% is a loan to the company?

A for-profit proposing a clinical trial is highly encouraged to secure or have a plan for securing a minimum of 50% of the total amount of funding at the time of submission of the research award. Reviewers will weigh that information as one of the criteria to consider. For-

profit entities are not eligible for grants, so any CIRM funds awarded for eligible activities are in the form of a loan.

Clinical Trial questions

14. Is an Investigator Brochure (Part G) required of all applicants requesting to conduct clinical trials?

CIRM would like a copy of the Investigator's Brochure that has been or will be submitted if it will be required by the FDA and/or your IRB.

15. We have a rather mature study that is in the process of filing an IND but we would like to obtain additional funding to enroll more patients. Do you think we can apply for this grant?

Each proposed project must meet at least one of the objectives stated in Section II of the RFA. For clinical trials, it is important that any CIRM-funded study be completed within the award period. For purposes of this RFA, a clinical study is considered complete upon completion of enrollment, database lock and initial assessment of outcomes of the primary and secondary study objectives.

CIRM recognizes that clinical studies may be at different stages (i.e. ready to initiate versus already underway) at the time of the application. An applicant is potentially eligible if your clinical trial is underway, but need additional funding.

16. Would we be eligible to apply if we have completed enrollment for Phase 1, but the trials have to be finished?

The answer to Question 15 above applies.

17. What if we plan to file an IND and then begin a trial in Year 3 of the project?

The answer to Question 15 above applies.

Grant review and awarding questions

18. Will the Grants Working Group for the Disease Team Research Awards be the same as that which reviewed the Planning Award applications?

There may be some overlap; however, because of the exception pathway, the therapeutic areas may change and we anticipate the GWG composition will not be the same.

19. Can for-profit organizations qualify for grants instead of loans?

No, for-profits entities would be funded through loans under this RFA.

20. Our timeline for IND submission has been accelerated since submitting the Planning Award. What are the implications for obtaining funding for IND-enabling activities that have already been completed?

CIRM will not reimburse for work that has already been completed before the application is approved. CIRM funds can be used for eligible post-approval activities up to 90 days prior to the start of the award period.

21. Can a for-profit hold more than one Disease Team award?

Yes but they require different Principal Investigators.

22. If I have one Planning Award, can I submit two proposals if we use different PIs?

No. The PI, scope, and project proposal of your Research Award application should be substantially the same as your Planning Award.

23. Will CIRM limit the number of awards to a for-profit company in this cycle?

CIRM does not have a target or maximum number of awards that will be made to for-profit entities. The decisions on awards will be made by the ICOC based on Grants Working Group review of the proposals received.